

**Sequential Transplantation of Umbilical Cord Blood Stem  
Cells and Islet Cells in Children and Adolescents with  
Monogenic Immunodeficiency Type 1 Diabetes Mellitus**

**2018-10-16**

## **Statistical Analysis Plan**

### 1. Data collection:

Management team: The patients will be followed up by a fixed team of senior doctors and nursing staffs in our center, and all the data will be collected by the same team.

Data collection: Baseline data, such as gender, age, birth weight, medical history and family history, fast plasma glucose, HbA1c, serum insulin concentration, serum C-peptide concentration, blood immunoglobulin, T lymphocyte subsets, interleukin-2 and so on will be collected when participants are diagnosed as monogenic immunodeficiency type 1 diabetes mellitus, occurrence of infection will be evaluated before and after the sequential transplantation of umbilical cord blood stem cells and islet cells. The follow-up will be carried out once every three months until three years after transplantation. The occurrence of infection will be documented according to caregivers and medical history at each visit. In the event of dropouts or withdrawals, the reasons for each missing value will be recorded.

### 2. Statistical analysis

2.1 Data recording: Data will be recorded in a purpose designed Excel sheet by the researchers.

2.2 All paper data will be stored in locked filing cabinets and electronic data will be password protected.

2.3 A statistician who is not affiliated with this study will perform the statistical analyses.

2.4 The comparison of the glycometabolism and immune function between before and

after the sequential transplantation of umbilical cord blood stem cells and islet cells, including fast plasma glucose, HbA1c, serum insulin concentration, serum C-peptide concentration and occurrence of infection will be analyzed using SPSS software. All of these indicators will be displayed as the mean, SD, and minimum and maximum values. One-way ANOVA will be used to examine significant differences for continuous variables. Two-sided p values less than 0.05 will be considered significant.